



# Financial Advocacy in Rare Diseases

*the Indian Scenario*

Supported by



October 2022

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# Executive Summary

## Aims

This study aimed to assess the awareness and accessibility of financial schemes for the rare disease community in India through surveys and stakeholder interviews to generate a report highlighting the current scenario in the country. The generated report will be widely published to benefit the rare disease community.

The project involves the following deliverables:

1. A comprehensive report containing a detailed analysis of the financial aid schemes available in the country and the challenges faced by the rare disease community in accessing these schemes generated through a combination of literature analyses, online surveys, and structured interviews with various stakeholder groups. This report will serve as an important resource to understand the current situation as it summarizes information on avenues to access financial aid for patients with rare diseases. It will be freely available for download from the IndoUSrare website and social channels and widely disseminated to educate the public.
2. A live-recorded webinar-style expert panel discussion on the awareness of the financial schemes available in India will be published on the IndoUSrare website.
3. Informational resources: A non-exhaustive list of financial resources available to rare disease patients in India will be published on the IndoUSrare website.

## Methods

Information was gathered through a combination of literature analyses, surveys (information requests), and stakeholder interviews. Two different types of information requests were designed and utilized to target various rare disease stakeholder types: 1) patients, patient advocates, or caregivers and 2) payors, grantors, or health professionals. Structured interviews were conducted using Zoom to capture the perspectives of diverse stakeholders on the accessibility and awareness of financial schemes. We received responses from almost 40 stakeholders who represented the patient community (patients, caregivers, and advocacy professionals) as well as several healthcare professionals, researchers, and grantors.

## Key highlights

1. All patient respondents were active members of the rare disease community in India, approximately 50% of whom were involved in advocacy efforts for their disease, largely as members of patient organizations. The health professionals reported that patient advocacy organizations also serve as important sources of disease-specific information.
2. More than half of the respondents had pediatric-onset rare diseases and had been diagnosed before the age of 10 years. Less than 20% of the rare diseases reported in this study were adult-onset type.
3. Financial well-being:
  1. Half of the patients surveyed did not have any approved treatment for their rare disease, and almost all those who had an approved therapy required expensive, long-term treatment.
  2. None of the surveyed patients reported receiving financial support from any source for diagnosis or treatment.

3. According to healthcare professionals, the two main reasons patients face financial hardships are 1) expenses due to high therapy costs and 2) searching for an accurate diagnosis.
4. Awareness of existing schemes:
  1. Most patients were unaware of government schemes for financial assistance for their rare diseases. We believe this is either because their rare disease was not included in the officially recognized list or because they were unsure of their eligibility due to socio-economic factors.
  2. Most reported a lack of private funding for specific rare diseases, including humanitarian access programs.
  3. Health professionals have identified the main gap in financial awareness as the ability to manage healthcare expenses and make sound healthcare decisions.
5. Access to insurance:
  1. Almost 95% of the patients were unable to access health insurance because of their rare diagnosis, and the remaining few were dependent on insurance schemes acquired prior to diagnosis.
  2. The claims mostly covered hospitalizations and surgeries, followed by medication expenses. The non-medical claims were mostly attributed to assistive devices.

## Recommendations

This study highlights that the main financial burden on families is related to accessing treatment. Various improvement measures have been suggested, including increasing the number of Centres of Excellence for rare diseases, increasing rare disease research to promote indigenous and affordable diagnostic and treatment options, and inclusion of rare diseases as an option for corporate social responsibility (CSR) donations.

Given the current scenario and resource-constrained nature of the country's healthcare system, investigators recommend an increased focus on improving the implementation of cost-effective screening and prevention programs launched by the government as part of the National Rare Disease Policy 2021. We advocate for and support better and more widespread programs on a national scale to improve public awareness of all the available financial aid schemes.

There is also a critical need to improve access to clinical trials as a care option for patients awaiting life-saving treatment in India. We believe that cross-border collaborative programs are key to accelerating access to early diagnosis and treatment of the nearly 70 million patients with rare diseases in the country.

# Aims and Objectives

This study aimed to assess the awareness and accessibility of financial schemes for the rare disease community in India to generate a report highlighting the current scenario in the country and to provide avenues for the dissemination of validated information that will benefit the rare disease community.

The main deliverables of this project are as follows.

1. A comprehensive report that includes a detailed analysis of the financial aid schemes available in the country and the challenges faced by the rare disease community in accessing these schemes through a combination of literature analyses, online surveys, and structured interviews with various stakeholder groups. This report will serve as a key resource for understanding the current situation and avenues to access financial aid for patients with rare diseases. It will be freely available for download from the IndoUSrare website, and social channels and will be widely disseminated in the public interest.
2. A web panel discussion on the awareness of the financial schemes available in India will be conducted.
3. Informational resources: A non-exhaustive list of financial resources available to rare disease patients in India will be made available on the IndoUSrare website.

# Introduction

Rare diseases affect very few individuals in the general population. Although the prevalence of rare diseases is low, they affect a considerable proportion of the global population. Today, nearly 11,000 distinct rare diseases have been identified, of which 95% are genetic in origin<sup>1</sup> Each disease has unique traits and shows heterogeneity other diseases. However, the myriad challenges faced by patients and their families share some commonalities. One of the biggest challenges they face is the financial burden, which is associated directly or indirectly with their struggles during different phases of life.

**Diagnosis:** The rarest diseases are genetic. However, despite advancements in the genetic toolbox, identifying disease-causing variants remains challenging. On average, it takes seven years or more<sup>1-3</sup> to obtain a diagnosis.<sup>1-3</sup> This diagnostic odyssey is predominantly attributed to ineffective diagnostic procedures, lack of available test kits, insufficient scientific knowledge to interpret the test results, functional characterization of the disease variant, and multiple and/or overlapping disease conditions<sup>3-5</sup>. Moreover, in third-world countries such as India, major barriers to efficient diagnosis include the lack of protocols for genome sequencing and analyses, few well-trained technical staff, poor infrastructure, and inadequate funding to support diagnostic labs<sup>6</sup>.

**Disease awareness and finding experts/specialists:** The next challenge faced by the rare disease community is the lack of awareness of their condition and knowledge of how to manage their symptoms. Medical practitioners are often not trained in dealing with symptoms or providing adequate care and do not provide up-to-date information regarding treatment strategies and support facilities for patients with rare diseases. Patients travel to specialized centers in different localities hoping to obtain better advice about their condition. This leads to further tests, physician fees, and travel expenses, all of which increase their expenditures. However, in most cases, these efforts fail, forcing patients to deal with their condition without a sufficient understanding of the disease.

**Financial difficulties for treatment:** Once a diagnosis is made, patients look forward to receiving effective treatment. However, only 5% of the rare diseases are treatable. In India, the Ministry of Health and Family Welfare (MoHFW) has estimated that if treatments exist, the cost will range from INR 1.8 to 17 million, which most cannot afford. In contrast, a study in the United States estimated that the total economic burden of 379 rare diseases was approximately USD 997 billion in 2019 and that it will continue to increase<sup>14</sup>.

An understanding of the expenditures encountered by patients with rare diseases and their families will help healthcare decision-makers make informed decisions on how to best allocate funds according to patient needs.

## National Policy for Rare Diseases:

MoHFW drafted the National Policy for Rare Diseases 2021<sup>17</sup> to support patients with rare diseases and their families. To date, the following support schemes have been implemented.

- Provision for funding up to **INR 200,000 under the Umbrella Scheme of Rashtriya Arogya Nidhi (RAN)** for the treatment of rare diseases that require one-time treatment<sup>21,25-26</sup>. Through this scheme, families below the poverty line and 40% of the population who are eligible under Ayushman Bharat-Pradhan Mantri Jan Arogya Yojana [a health assurance scheme that aims to provide a health cover of Rs. 5 lakhs per family per year for secondary and tertiary care hospitalization to poor and vulnerable families] can benefit from government tertiary hospitals<sup>14,17-18</sup>.



- Support of up to INR 500,000 for any rare disease category toward treatment at any Centre of Excellence outside RAN is also available<sup>18</sup>.

*Despite the implementation of the schemes mentioned above, a news report in May 2022 from Kerala voiced disappointment: "This provision would have been beneficial for a big community of patients suffering from all sorts of rare diseases had it not been for the lack of a Centre of Excellence in the state." Several people from Kerala were unable to become beneficiaries of the schemes as their state did not have a Centre of Excellence<sup>19</sup>.*

- State government support can be utilized for low-cost treatments such as special diets and hormonal treatments.
- In addition, the government has endeavored to create an alternative funding mechanisms by setting up a digital platform for voluntary crowdfunding to treat individuals, which can be accessed through the Centre of Excellence.

*When the draft was implemented, several patients' families were delighted to have their loved ones with a rare disease get registered in the portal. However, a father of a 9-year-old boy affected by mucopolysaccharidosis-1 (MPS-1), reported to be a public bus conductor, was unable to have his son registered in the portal because doing so would require a new round of tests and investigations that would cost INR 60,000–70,000<sup>20</sup>*

# Methodology

To understand the accessibility and awareness of financial schemes among rare disease communities in India the following goals are proposed:

Goal 1. Determine the availability and accessibility of various schemes for financial assistance for rare diseases.

Goal 2. Create an educational resource based on the findings.

Goal 3. Generate awareness of available financial schemes among rare disease communities in India.

To achieve these goals, we employed a multi-pronged strategy consisting of the following three methodologies:

1. **Information requests and surveys:** Two types of information request were selected by considering all rare disease stakeholders.

Information requests include the following:

a. Information requests for patients, patient advocates, and caregivers consisting of 25 questions, which were categorized into three sections:

i. General information: This section collects general information pertaining to the disease name, relationship with the patient, gender, and involvement in patient advocacy activities.

ii. Diagnosis and treatment of disease: This section collects data on the age at disease onset, time taken for diagnosis, total costs incurred for diagnosis, priority ratings of the families of patients with rare diseases, and the type(s) of treatment(s) available.

iii. Financial well-being: This section collects data on the obstacles families face in accessing healthcare and its affordability, the existing mode(s) of communication to learn about financial schemes, and, in general, seeks to understand the financial well-being of the respondent.

b. Information requests for payors, grantors, and health professionals, which include the following:

i. General information: This section collects data on all general information about the respondents, such as their name, email address, postal code, profession, name, and organization type.

ii. Diagnosis and treatment: This section was designed mainly to understand healthcare professionals' opinions, specifically pertaining to their experience with a particular disease, and to understand whether they faced any specific hurdles related to organizational finances. It includes questions such as the average time to reach a diagnosis, type(s) of treatment available, priorities of families of individuals with rare diseases, and maximum point of financial strain felt by the families.

iii. Financial well-being: This section was designed to gain insights into the patient's financial situation from the perspective of a healthcare professional. The questions included roadblocks faced by families of persons with rare diseases in access and affordability of health care, details on available financial aid, time required to access the financial scheme, financial hardships faced, and the reasons for the same.



We contacted approximately 150 rare disease stakeholders and obtained a total of 32 responses. This survey was conducted to cover two goals: 1) to determine the availability and accessibility of various financial assistance schemes for rare diseases and 2) to create an educational resource based on our findings from information requests.

2. **Structured Interviews:** We conducted online structured interviews lasting approximately 40–60 minutes with the aim of capturing first-hand information about individual perspectives on accessibility and awareness of financial schemes, consisting of the following two types:
  - a. Interviews with the patient or patient advocate
  - b. Interviews with rare disease healthcare professionals

Ten interviews were conducted with seven patient/patient advocates and three healthcare professionals. These interviews were conducted to cover two goals: 1) to determine the availability and accessibility of various schemes for financial assistance for rare diseases and 2) to create an educational resource based on our findings.

## Study Findings

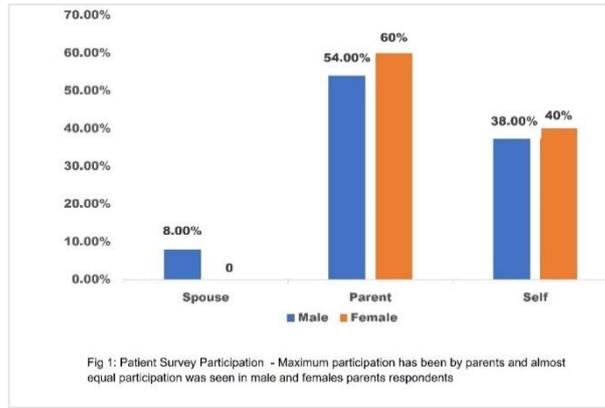
Families of patients with rare diseases in India face enormous challenges. The exorbitant costs and lack of easy access to treatment exert a considerable financial burden on them, often wiping out entire family life savings. There is an enormous dependence on imported drugs for rare disease treatment, which increases the cost of such treatments. Moreover, overheads, such as loss of caregiver jobs, travel to hospitals, supplementary medicines, and special diets, are additional expenditures that receive little consideration.

### Findings from the patients, patient advocates, and caregiver information requests

#### 1. General information

##### Patient demographics

The majority of the survey participants were parents, of which 54% were male and 60% were female. Patient participation comprised 40% females and 38% males, and the representation of male partners was only 8% compared to female partners with representation.

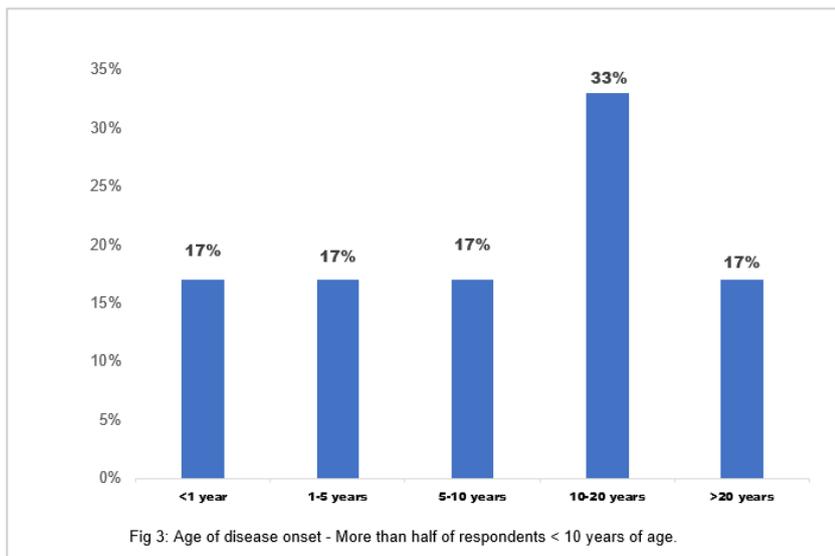


### Patient advocacy activities

More than half of the patients, patient families, and caregivers were actively involved in patient advocacy activities. Approximately 62% of the patients were involved in advocacy activities, while 38% of the patients/caregivers were not associated with any patient advocacy groups (Fig. 2).

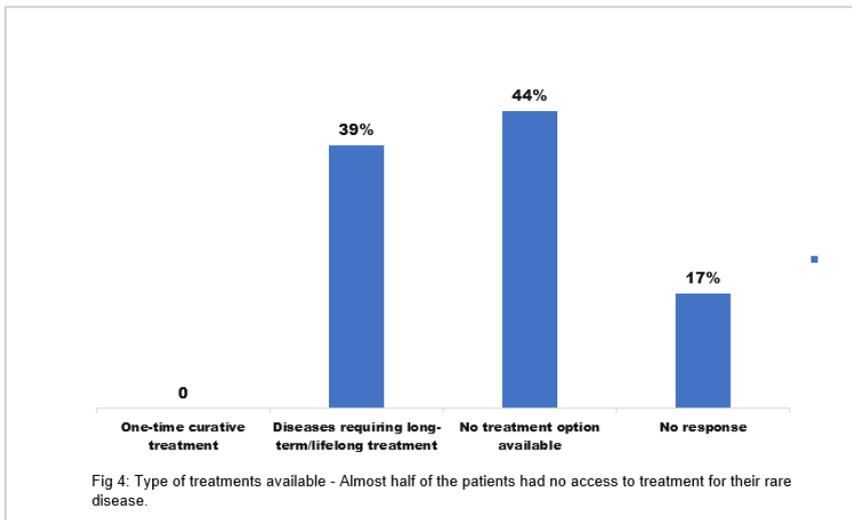
## 2. Diagnosis and treatment of disease

### Disease onset age



From the survey, it was observed that 51% of the cases had pediatric disease onset, with an age range of less than 1–10 years. The median age at disease onset was 9 years, and the average age was 3.6 years (Fig. 3).

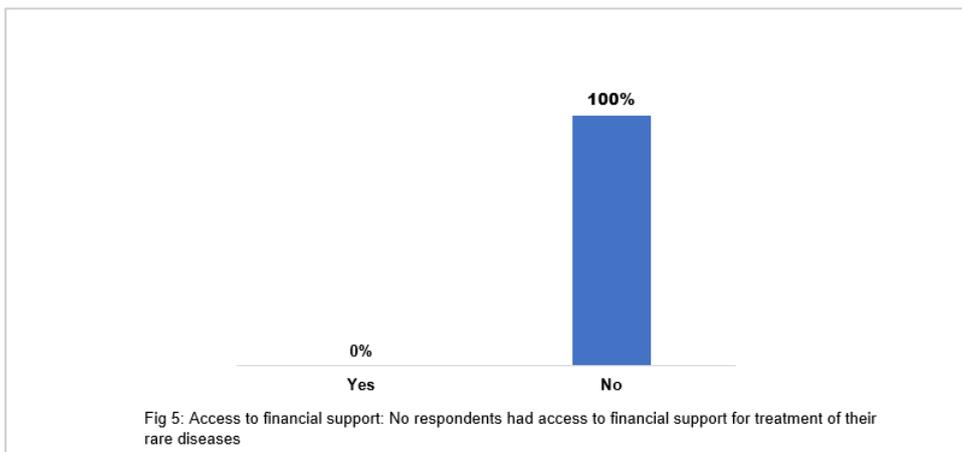
## Treatment availability



Almost half of the patients had no available treatment options for rare diseases. Approximately 44% of the patients with rare diseases had no access to treatment, and 39% required high-cost and long-term/lifelong treatment (Fig. 4).

## 3. Financial well-being

### Access to financial support



All respondents said that they were not provided any financial support during their diagnosis and treatment journey (Fig. 5).

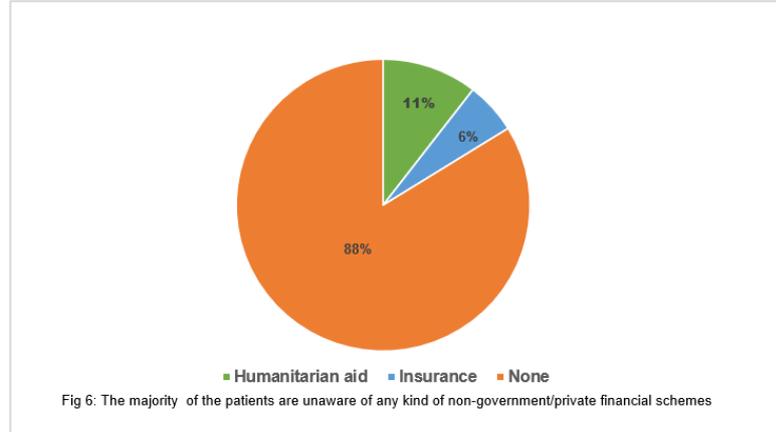
## Awareness of government and non-government financial schemes

Awareness of Government Financial Schemes	
None	83%
Wheelchair and pensions for disabled	5.50%
Rare disease treatment one time up to INR 500000	5.50%
State Government schemes, Genomics, and other omics tools for Enabling Medical Decision (GOMED) under AIIMS	5.50%

Table 1: The majority of respondents were unaware of any kind of government financial schemes.

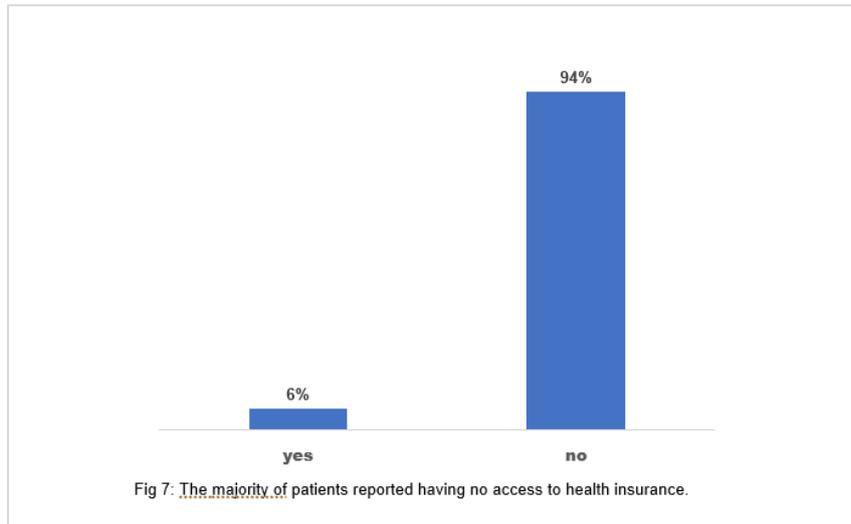
Most respondents were unaware of any government financial scheme. Only 6% of participants were aware of schemes such as wheelchairs and pensions for the disabled, one-time rare disease treatment (up to INR 50,00,000), and state government schemes under the All India Institute of Medical Sciences (Table 1).

## Awareness of non-governmental/private financial schemes

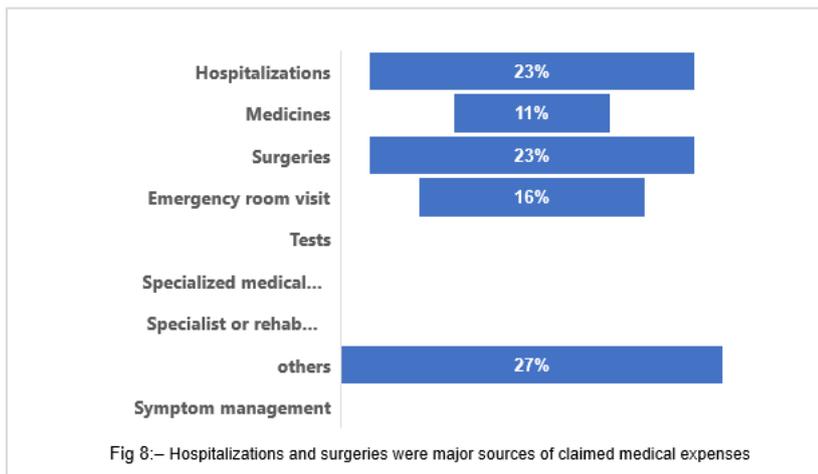


Of the respondents, 88% were unaware of any private financial scheme, 11% reported being aware of humanitarian aid, and 5.5% were aware of insurance schemes.

## Patient Access to health insurance

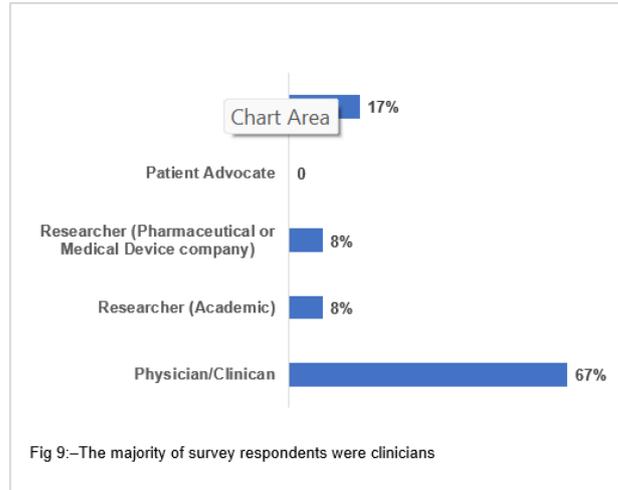


The majority (94%) of patients responded that they were ineligible to apply for any kind of health insurance because of their health condition, and that they had to pay out-of-pocket for most treatment-related expenses. Only 6% of the respondents had health insurance, with the insurance policy having been purchased prior to their rare disease diagnosis (Fig. 7).

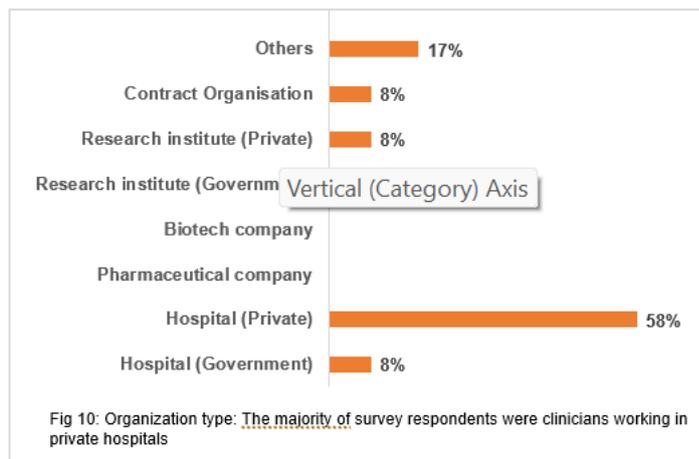


A considerable proportion (23%) of patients with rare diseases have received approval for hospitalization and surgery. Approximately 16% of the patients were able to claim emergency room visits, 11% claimed medicines, and 27% claimed other medical expenses. The reported non-medical expenses included education and medical devices.

## Information from the payors, grantors, and health professional surveys

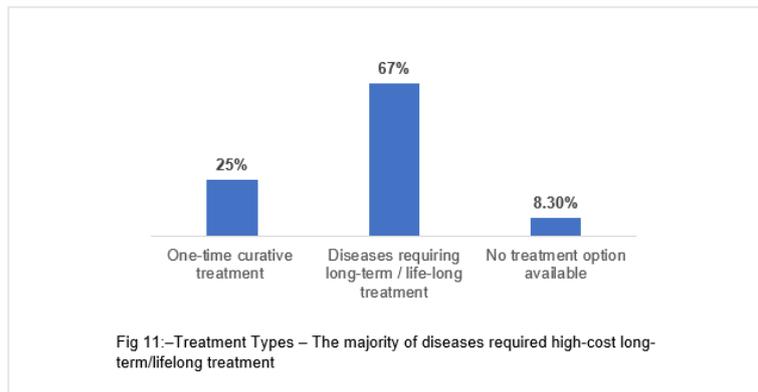


The majority of survey respondents (67%) were clinicians, followed by pharmaceutical or academic researchers (8%) and other professions (17%) (Fig. 9).



Most participants (58%) were clinicians employed in the private hospital sector, followed by 8% from government hospitals, contract organizations, and research institutes, and 17% from other sectors (Fig. 10).

## Treatment types

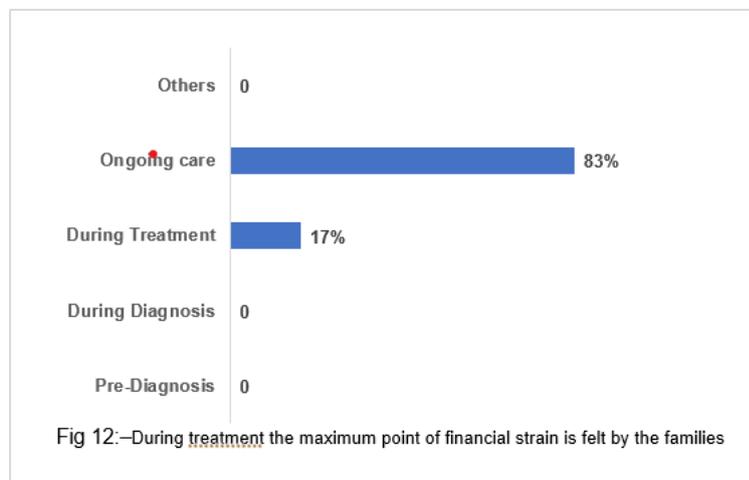


The majority (67%) of healthcare professionals reported that rare diseases required long-term or lifelong treatment, while 25% reported that there were rare diseases that required one-time curative treatment. Only 8% reported having no treatment options (Fig. 11).

According to healthcare professionals, 58% of patients required high-cost lifelong treatment, and 42% reported needing low-cost, lifelong treatment.

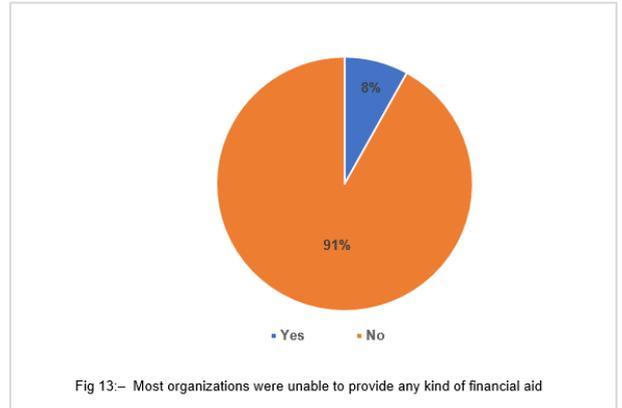
## Financial well-being

During the survey, 83% of health professionals reported that the maximum financial strain on the patients was during treatment rather than diagnosis, and 17% reported that the maximum financial strain was felt during ongoing care (Fig. 12).



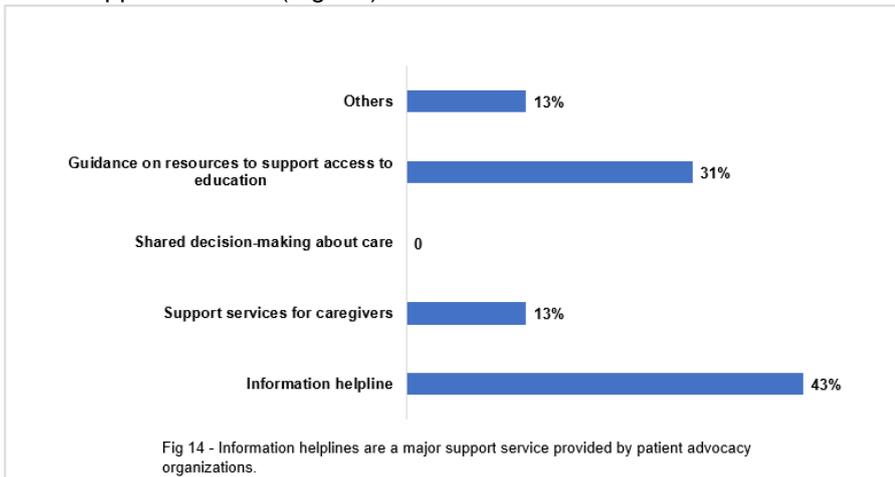
## Financial aid Provided by Organizations

During the survey, 91% of the health professionals responded that they did not provide financial aid to rare disease patients, whereas 8% responded that they did provide financial aid (Fig. 13).

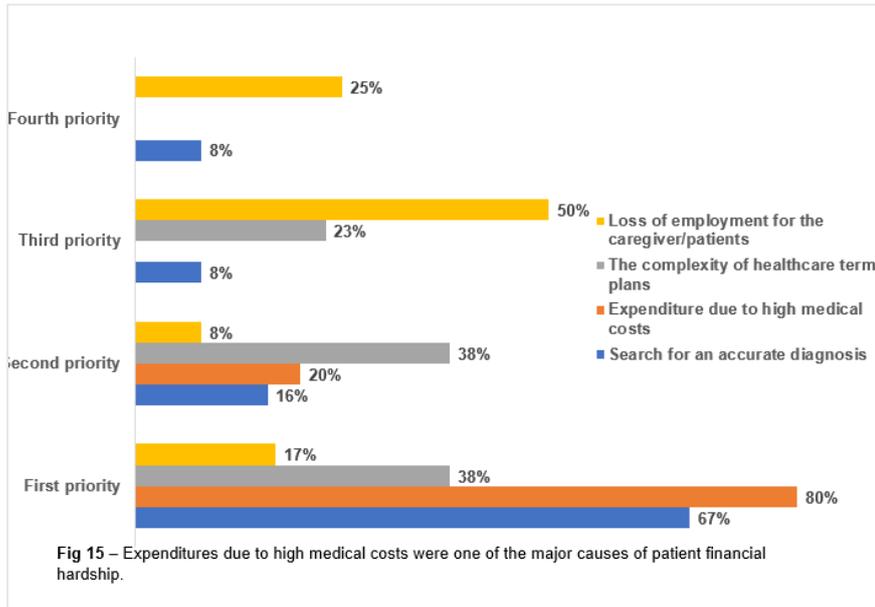


## Support services provided by patient advocacy organizations

When asked about their opinions on the types of services provided by patient advocacy organizations, 43% of professionals reported that they offered information helplines, 13% acted as support services, 31% provided guidance on how to access information about a particular rare disease, and 13% provided other support services (Fig. 14).



## Major Financial Hardships Faced by Patients



The health professionals were asked to identify the reasons patients faced financial hardships. We categorized the responses into four categories (first to fourth priority), where the first priority denotes major hardships (Fig. 15).

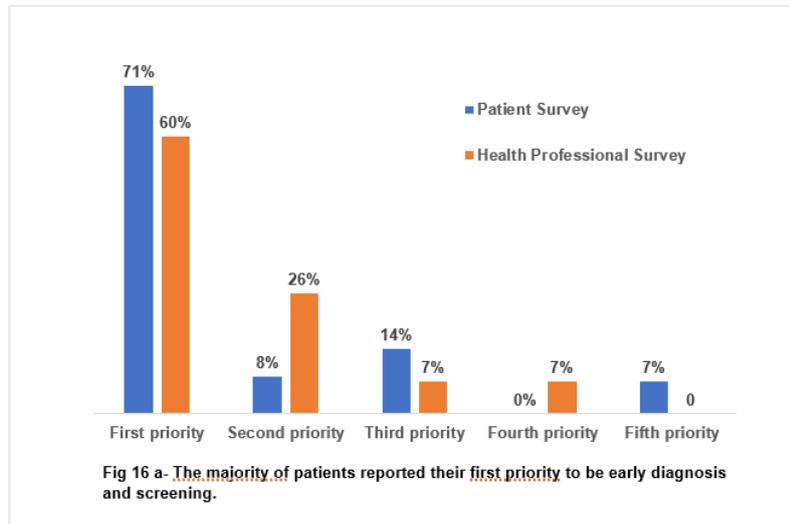
High medical costs were cited by 80% of the respondents as the most common cause of financial hardship. Often in India, where many patients come from middle-income families, medical bills take a toll on savings. Similarly, 67% of the respondents identified the high costs incurred while trying to obtain an accurate diagnosis as a major hardship. Loss of employment for caregivers and/or patients has emerged as another factor contributing to families' financial burden. Taking a step back, Fig. 1 shows that the majority of respondents were parents. In India, it is not uncommon for mothers to choose to support the daily routines of the rest of the family members, whereas fathers act as the primary breadwinners for the family. Supporting the entire family through a single source of income is generally challenging, particularly when there is a family member with a rare disease. Moreover, not all patients with rare diseases have the potential to be employed, further constraining them financially.

### Comparison of responses of health professional and patient surveys

#### 1. Priorities of Rare Disease Families

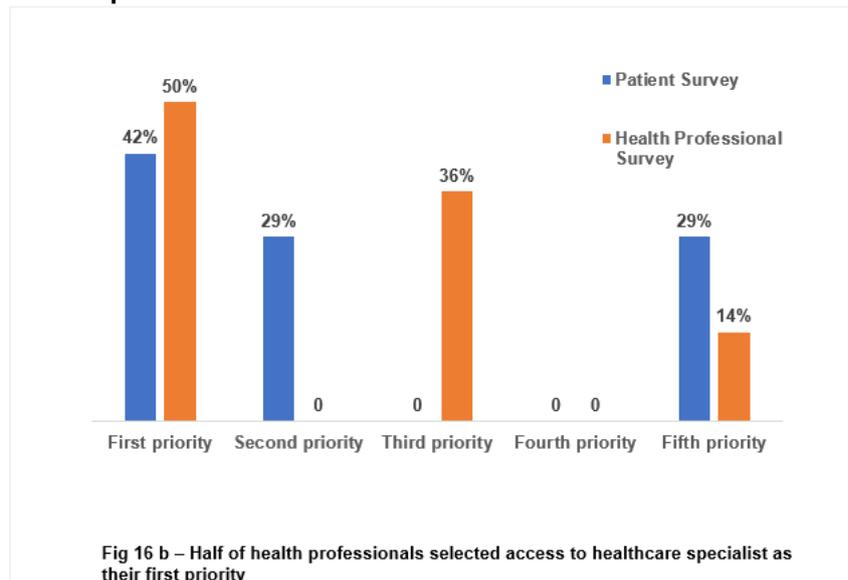
We analyzed several factors along the rare disease families' diagnosis and treatment journey to provide a better understanding of the priorities and hurdles they may face.

## Early Diagnosis or Screening for Disease



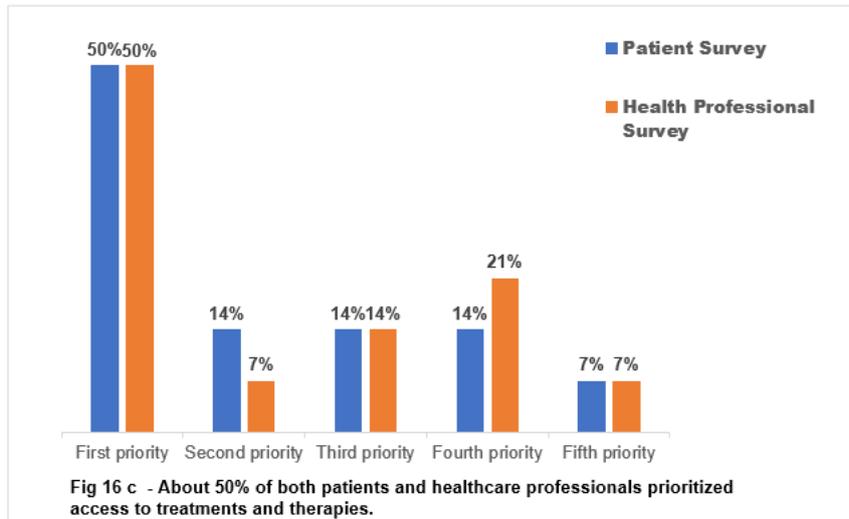
Both patients and health professionals indicated early diagnosis and screening as primary concerns (71% of patients and 60% of health professionals), indicating that early diagnosis is a critical need for patients and families (Fig. 16a).

## Access to Healthcare Specialists



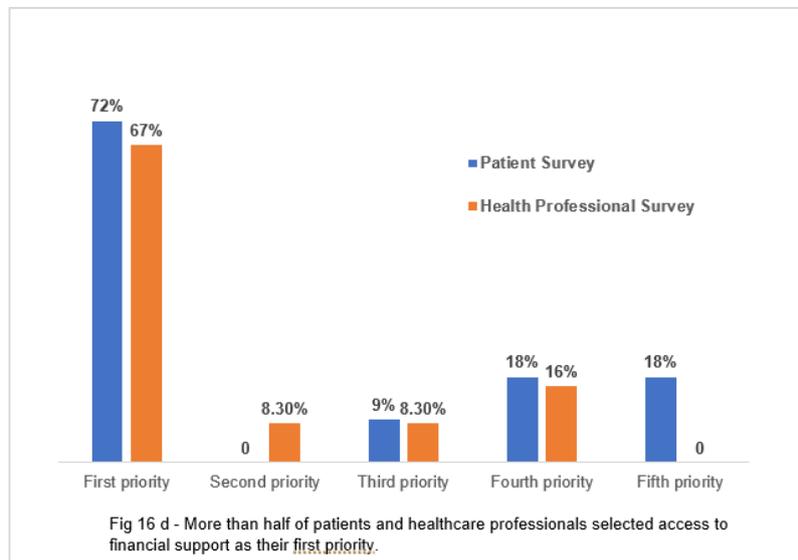
Half of healthcare specialists and 42% of patients indicated that access to healthcare specialists was their first priority. In contrast, almost 30% of patients did not consider this a concern (Fig. 16b).

## Access to Treatments and Therapies



Almost half of patients and health professionals stated that access to treatment and therapies was their first priority, demonstrating that both types of stakeholders believe that access to treatment and therapy is an important priority for families with rare diseases (Fig. 17c).

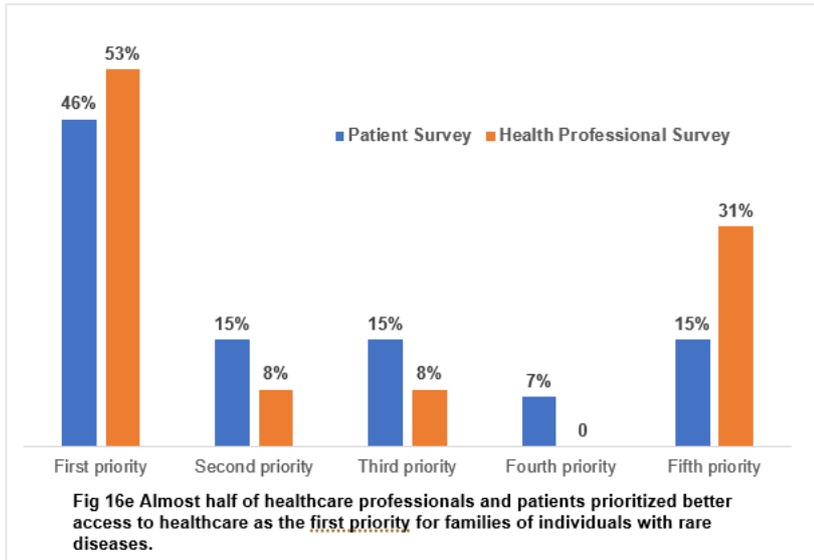
## Access to Financial Support



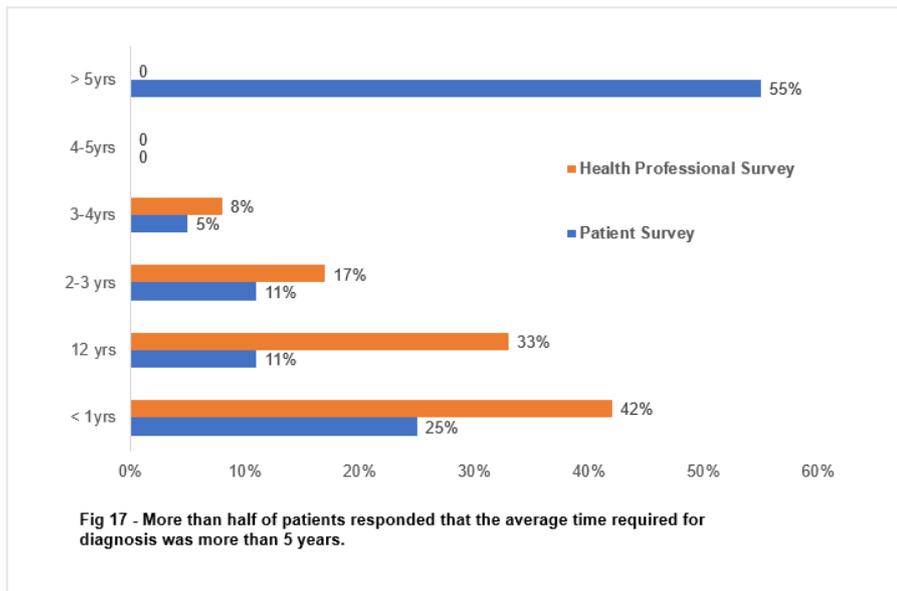
Access to financial support was identified as a major concern by more than half of patients and health professionals surveyed (Fig. 16d).

## Access to Better Healthcare

Almost half of healthcare professionals and patients prioritized better access to healthcare as one of the first priorities for families of patients with rare diseases.



## 2. Average Time Required for Diagnosis



Rare diseases cannot be diagnosed as quickly as other diseases, and individuals with diagnostic journeys for rare diseases are significantly prolonged owing to the unavailability of testing and limited access to specialists. Additionally, there is a lack of adequate screening, especially in the remote areas of the country (Fig. 17).

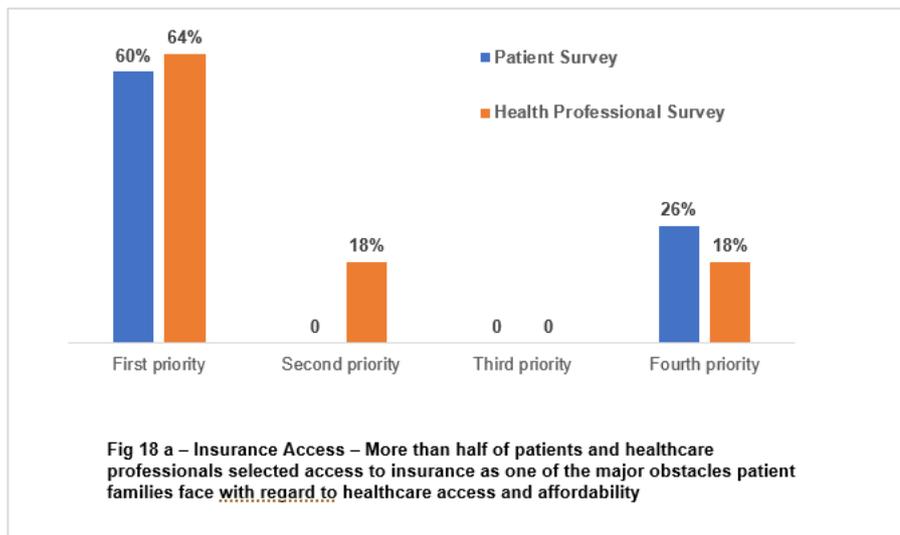
Without distinguishing between diseases, the response to the question regarding the average time required for diagnosis highlighted a notable discrepancy. The results indicate that 55% of patients reported that it takes more than five years to obtain a complete diagnosis. However, 42% and 33% of health professionals stated that a full diagnosis can be made within a year and one to two years, respectively. Only 17% of the patients reported less than 1 year of diagnosis. There was also a difference in opinion regarding the median age at diagnosis, as summarized in Table 2.

### 3. Median and Average Age of Diagnosis

Type of Survey	Medians age (yrs)	Average age (yrs)
Healthcare professional survey	3.5	2.3
Patient survey	2	3.6

Table 2 – Healthcare professionals' opinions of the average age of diagnosis was less than half than that of patients

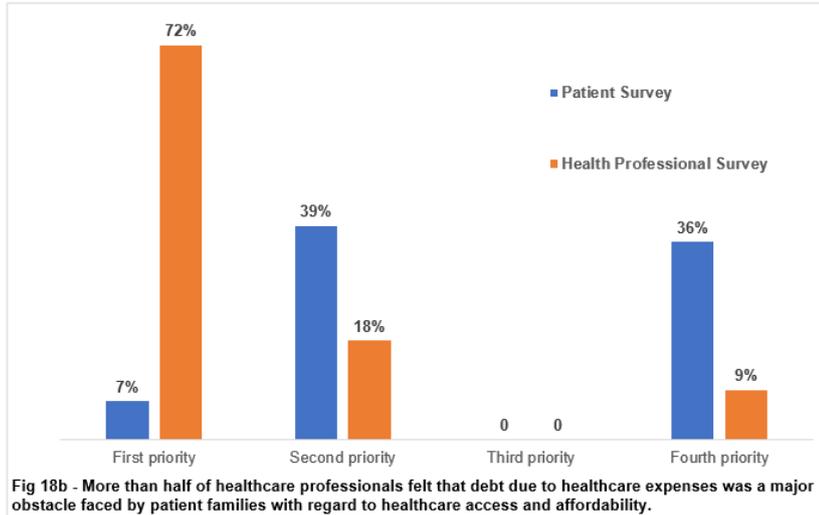
### 4. Obstacles Faced by Patient Families in Healthcare Access and Affordability Insurance Access



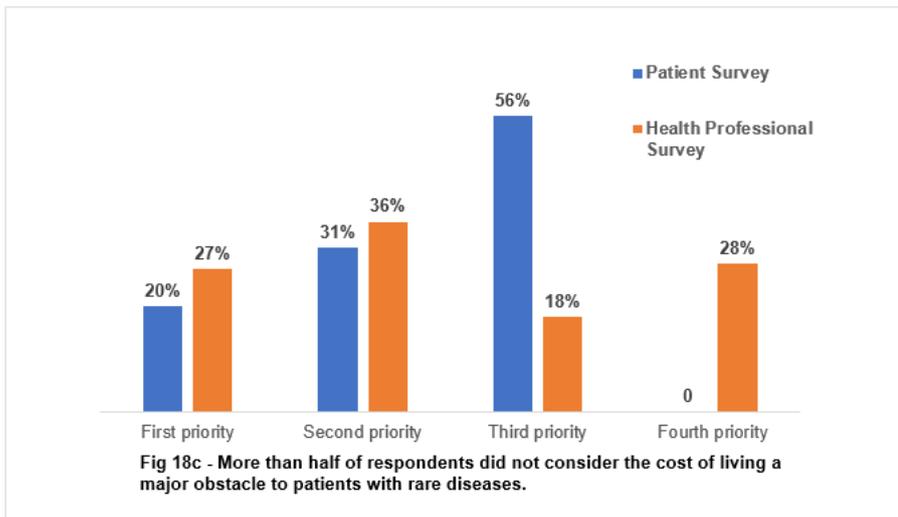
Approximately 60% of healthcare professionals and patients reported insurance access as the primary obstacle to accessing healthcare (Fig. 18a).

### Debt Due to Healthcare Expenses

Most healthcare professionals (72%) reported debt due to healthcare expenses as the first obstacle to accessing healthcare, while 39% of patients rated it as the second obstacle. (Fig. 18b)



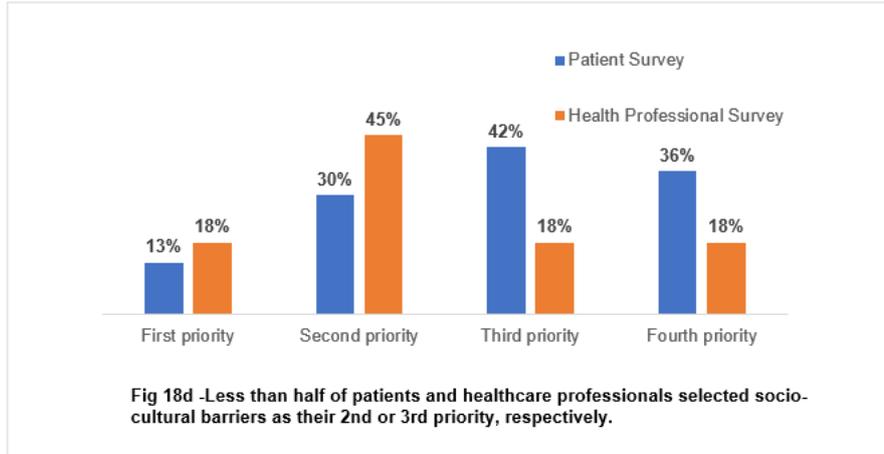
### Cost of Living



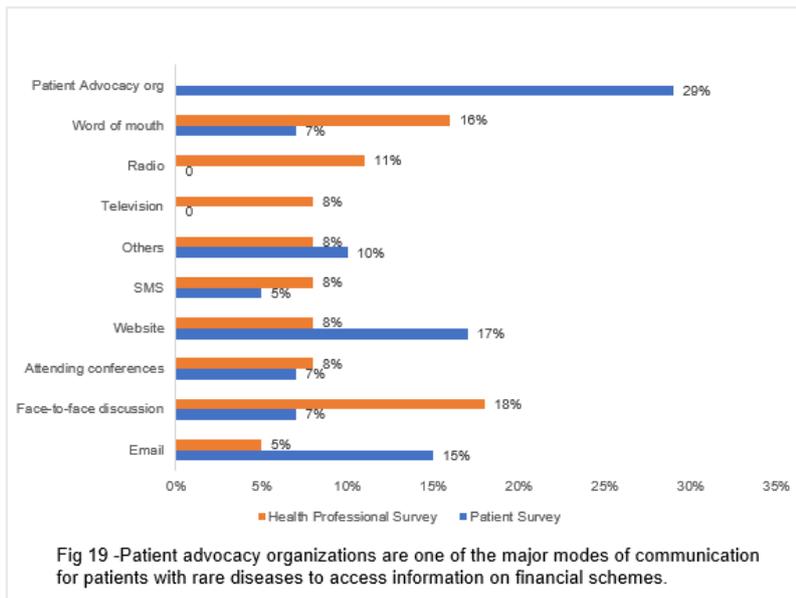
More than half of respondents did not consider the cost of living a major obstacle to patients with rare diseases. Approximately 56% of patients rated the cost of living as the third highest obstacle to accessing healthcare, compared to 36% of health professionals who rated it as the second highest obstacle (Fig. 18c).

### Socio-cultural Barriers

Less than half of patients (42%) and health professionals (45%) chose socio-cultural barriers as a third and second priority, respectively, which shows that socio-cultural barriers are one of the lowest priorities with regard to healthcare access and affordability (Fig. 19d).



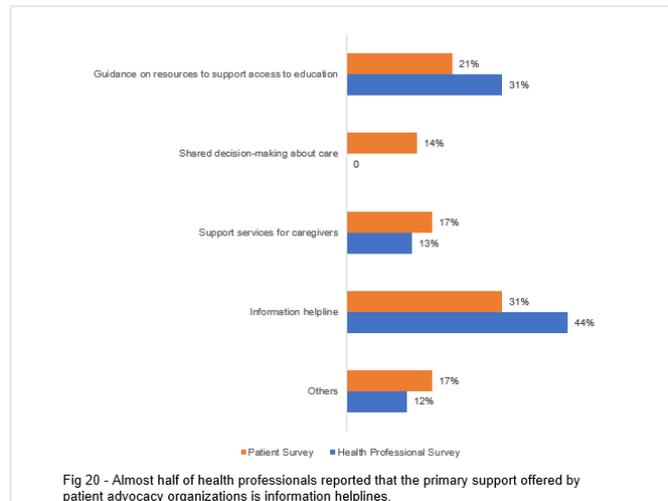
### 5. Modes of Communication about Existing Financial Schemes



Despite the release of the National Rare Disease Policy, there are no clear guidelines on implementing government support schemes. Many families remain unaware of their healthcare rights and are unsure of their eligibility status. According to the patient survey results, the most important source of communication was patient advocacy organizations (29%), followed by websites (17%) and email (15%). By contrast, health professionals selected face-to-face discussion (18%) as the most

important means of communication, followed by word of mouth (16%) and newspapers/radio (11%) (Fig. 20).

## 6. Type of Support Provided by Patient Advocacy Organizations



Patient advocacy organizations play a major role in supporting families with rare diseases in their journey from diagnosis to treatment and management of the disease.

In our survey, patient families and healthcare professionals reported that the major support provided by these organizations was delivered through information helplines (Fig. 21).

## Interview Findings

Stakeholder interviews were conducted to assess the awareness and accessibility of financial schemes and gather expert opinions from various perspectives, including patients, caregivers, patient advocates, and healthcare professionals. Ten interviews were conducted with seven patients/patient advocates/caregivers and three healthcare professionals (Table 3).

The various stakeholders who were interviewed belonged to the following categories:

S. No	Stakeholder	Number
1	Patients	2
2	Patient Advocates	5
3	Healthcare Professional (Academic)	1
4	Healthcare Professional (Private)	2

Table 3 – Participation of the number of stakeholders in the interview

The key themes to emerge from the interviews were as follows:

**1. Time required for diagnosis and costs involved.**

The overall time taken for diagnosis ranged from one to five years. Only two out of 18 respondent diagnoses were relatively shorter, while others faced issues confirming the subtype of the disease. Most of the interviewees highlighted that there was a delay in diagnosis as there was no standard protocol followed for the diagnosis of rare diseases and few appropriate laboratory facilities.

**2. Type of guidance received from the healthcare practitioner regarding the costs involved in the diagnosis and treatment of the disease**

Most interviewees said that when they received their diagnosis, they did not receive any guidance on the costs involved as there was no treatment option at that point. Some patients received advice on the costs involved and the latest clinical trials. Some highlighted that doctors said there was no hope for medicine, and even mentioned possible life expectancy.

**3. Costs involved in treatment and management of disease**

Interviewees' responses varied based on the disease, ranging from 120 USD/month to 1,500 USD/month. Most rare diseases do not have any treatment, but for diseases such as Gaucher disease, the treatment cost is approximately USD 36,000–50,000. Some diseases have available gene therapy treatments, which costs approximately 2,500,000 USD.

**Key Highlight 1**

***“Correct data was there but we were not able to make a diagnosis due to a lack of experience in detecting the defect by the doctor’s side.”***

Mr. Shreyansh Srivastava (Patient Facioscapulohumeral Muscular Dystrophy)

**Key Highlight 2**

***“Doctors’ approaches to patients should be more empathetic.”***

Mr. Vasanth Rao (spouse and patient advocate of Motor Neurone Disease)

**Key Highlight 3**

***“Just because my child has a DMD gene, he should not be treated unequally by health insurance companies.”***

Mr. Ambrish Kapadia (father and patient advocate of Duchenne muscular dystrophy disease)

#### 4. Access to financial assistance

##### Key Highlight 4

***“Transparency from government is expected and patients should know what kind of financial and medical aid is available.”***

Mrs. Anita Santosh  
(mother and patient  
advocate of Duchenne  
muscular dystrophy)

All interviewees mentioned that they did not have access to any kind of financial assistance. Some have not tried to apply for any kind of health insurance as they think they are ineligible for financial help due to their sound financial status. Some respondents had health insurance before they were diagnosed with a rare disease, which covered a few of their hospitalizations and medications. The interviewees wanted to highlight the fact that none of the rare diseases were covered by health insurance companies, which became an added burden to their families.

#### 5. Types of government/non-government schemes available for patients

Most interviewees were unaware of available schemes for patients with rare diseases. They also did not know how to implement these schemes. Some of them were aware of schemes such as RAN, but mentioned that the access procedure was extremely tedious as all patients had to access these schemes through a national Centre of Excellence. Some respondents were aware of humanitarian access programs, which are not a sustainable option.

##### Key Highlight 5

***“Crowdfunding needs to be monitored.”***

Mr. Iftikhar Md Zia (father  
and patient advocate of  
ataxia disease)

#### 6. Opinions on crowdfunding

Most respondents were aware of crowdfunding. According to some, crowdfunding is suitable for diseases that have treatment options but is not a sustainable option. The success rate of crowdfunding is low and the access process is quite complex. The government does not efficiently monitor crowdfunding.

##### Key Highlight 6

***“Governments should invest in indigenous drug development for rare diseases to reduce the costs of drugs.”***

Mrs. Anita Santosh  
(mother and patient  
advocate of Duchenne  
muscular dystrophy)

#### 7. Types of support expected from the government

- Rare diseases should be part of either the National Health Mission or State Health Mission.
- There should be no bifurcation between government and private hospitals; they must work in a cohesive manner. Government-led laboratories should be able to operate in private hospitals.
- The government should reduce procedural approval time, take a more transparent approach to research, and encourage funding for scientists working in rare disease fields.
- They should also pass laws to ensure that all patients with rare diseases are covered by health insurance.

# Conclusion

1. All respondents in the patient community were well informed, with approximately 50% being actively involved in advocacy efforts for their disease, mostly as members of a patient organization. Health professionals also reported that patient advocacy organizations serve as important sources of disease-specific information.
2. More than half of the respondents had pediatric-onset rare diseases and most were diagnosed before the age of 10 years. Less than 20% of the rare diseases were adult-onset diseases.
3. Financial well-being:
  - a. Half of the patients surveyed did not have any approved treatment for their rare diseases, and diseases with treatment options required high-cost, long-term treatment.
  - b. None of the surveyed patients reported receiving financial support from any source for diagnosis or treatment.
  - c. According to healthcare professionals, expenditures due to high costs and the search for an accurate diagnosis are the main reasons patients face financial hardship.
4. Awareness of existing schemes:
  - a. Most patients were unaware of government schemes for financial assistance for their rare diseases. We believe this is either because their rare disease was not included in the officially recognized list or because they were unsure of their eligibility due to socio-economic factors.
  - b. Most reported a lack of private funding for specific rare diseases, including humanitarian access programs.
  - c. Health professionals identified the main gap in financial awareness as the ability to manage healthcare expenses and make sound healthcare decisions.
5. Access to insurance:
  - a. Almost 95% of the patients with rare diseases were unable to access health insurance because of their rare diagnosis; the remaining few were dependent on insurance schemes acquired prior to diagnosis.
  - b. The claims mostly covered hospitalizations and surgeries, followed by medication expenses. Non-medical claims are mostly attributed to assistive devices.
6. Interview findings:
  - a. Crowdfunding is suitable for raising funds for diseases that have treatment options but might not be a sustainable option.
  - b. Most of the interviewees were unaware of available schemes for patients with rare diseases.
  - c. Rare diseases should be included in the National Health Mission (NHM) or national NHM.
  - d. There was a request to improve the process of accessing funds by making them more systematic.
  - e. The need to develop indigenous drug development was highlighted.
  - f. Crowdfunding should be monitored by the government.
  - g. Health insurance should be available for all.

# Recommendations

This study highlights that the maximum financial burden on families is centered on their access to treatment. Various methods of improvement have been suggested, including increasing the number of Centres of Excellence for rare diseases, increasing rare disease research to promote indigenous and affordable diagnostic and treatment options, and including rare diseases as an option for CSR donations.

Given the current scenario and resource-constrained nature of the country's healthcare system, the authors recommend an increased focus on improving the implementation of cost-effective screening and prevention programs launched by the government as part of the 2021 National Rare Disease Policy. We recommend better and more widespread programs at a national scale to improve awareness of all the available financial aid schemes.

IndoUSrare compiled a non-exhaustive list of financial resources available to rare disease patients in India, which is available at the link below. The resource list will be updated as information on more schemes is acquired (<https://www.indousrare.org/financialadvocacy/>).

Disease management plays a significant role in rare diseases as 95% of patients with rare diseases do not receive treatment. Strengthening alternative treatment options, such as physiotherapy and yoga, might be a viable option for long-term disease management. Physiotherapy sessions are expensive and unaffordable for all socio-economic groups. Hence, we recommend strengthening physiotherapy government-run clinics that can provide subsidies. Even special schemes/incentives for physiotherapy can be provided to patients with rare diseases.

There is also a critical need to improve access to clinical trials as a care option for patients awaiting life-saving treatment in India. We believe that cross-border collaborative programs are key to accelerating access to early diagnosis and treatment of nearly 70 million patients with rare diseases in the country.

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## Authors List

1. Ramya T Karur MSc. IndoUSrare Project Associate
2. Ms. Preethi Krishnaraj Max Planck Institute for Heart and Lung Research, Germany Pursuing Ph.D. Intern in IndoUSrare
3. Nisha Venugopal, PhD. IndoUSrare Research Scientist, and Program Manager
4. Reena Kartha, MS, PhD. Board member of IndoUSrare and Assistant Professor and Associate Director of Center for Orphan Drug Research, University of Minnesota.
5. Narayanan Govindarajan, MS. Board Member of IndoUSrare and Leader in Bigdata and analytics technologies.
6. Harsha Rajasimha, MS, Ph.D. Founder and Chairman of IndoUSrare